VIEWPOINT

Comparative Effectiveness Research: Promise or Peril for Biotechnology?

BY SHANNON DOYLE

In his frequent calls for healthcare reform, President Obama has insisted on improving the quality of healthcare for all Americans as well as reducing its cost. Daunting as though that task may be, the evidence suggests that as much as one third of healthcare dollars are wasted on treatments that neither improve health nor increase patient satisfaction (Brownlee 2007). Theoretically, healthcare spending could be cut by nearly $1 trillion per year without adversely affecting patient outcomes. The challenge for policymakers is to identify inefficiencies and then change the behaviors that promulgate them without limiting those aspects of the system that work well and embody healthcare values.

The biotechnology industry is aware of the rapidly changing healthcare landscape, particularly the controversial Comparative Effectiveness Research (CER) program that is called for under the American Recovery and Reinvestment Act (ARRA) of 2009, which allocates $1 billion for CER. Although that amount represents a relatively small fraction of the ARRA economic stimulus package, the money dedicated to CER has triggered a strong response from policymakers, provider organizations, patient groups, healthcare industry representatives, and other stakeholders. Most of them agree that the notion of comparing the effectiveness of drugs and medical treatments is a first step in the right direction for healthcare reform. Who can argue with efforts to distinguish products and practices that work and are of value to patients from those that do not achieve the desired outcomes? The ultimate impact of CER on the many stakeholders, however, remains uncertain.

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The Dilemma

The primary debate about CER is whether the financial cost of drugs and clinical interventions should be considered in publicly funded studies used to make coverage decisions.

ARRA does not explicitly address the issue of cost. The wording in the Senate version of the bill contained the term "comparative clinical effectiveness" to ensure that research would be strictly focused on patient outcomes. Ultimately, however, the wording in the House version of the bill was used, and the crucial word "clinical" was dropped. As it now stands, ARRA does not mandate cost considerations and underscores the role of CER in investigating the "clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health conditions" (ARRA 2009). Nevertheless, cost analyses may be included in the future.

Two Scenarios

Because of the ambiguous wording of the ARRA, CER is precariously poised between two different trajectories.

In one scenario, the resulting research will be used exclusively as a means to inform patients and physicians about the relative clinical effectiveness of therapeutics, procedures, and devices. Equipped with more and better information, patients and payers ideally would be able to avoid wasting valuable time, resources, and money on ineffective and useless treatment modalities.

The alternative route that CER could take is one that involves incorporating cost analyses in some capacity. The danger in considering cost when evaluating effectiveness is the possibility that payers, whether public or private, may decide to deny coverage for treatments that are not deemed cost-effective according to a certain federal standard. Comparative effectiveness procedures adopted by other countries that also include cost considerations illustrate this concern. An example is the United Kingdom's National Institute for Health and Clinical Excellence (NICE) — a CER entity that includes cost in its studies and recommendations — which denied coverage for the anti-tumor necrosis factor drug abatacept (Orencia) to patients with rheumatoid arthritis because it was deemed too expensive whatever its clinical benefits may be (Kahn 2007). Biotechnology companies in the United States will need to prepare for

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this type of situation because it may become common as CER expands.

In a statement to the Federal Coordinating Council for CER, the Biotechnology Industry Organization (BIO) voiced its concern that "Comparative effectiveness information may be used strictly as a means to contain costs, rather than deliver health care by improving patient outcomes" (BIO 2009). If the evolving American CER structure becomes focused on cost-reduction objectives rather than evaluating and improving clinical outcomes, the biotechnology industry may be vulnerable.

Even without the apprehension over cost analyses, biotechnology products — particularly those that cater to small subpopulations that might be overlooked in large studies — could become incidental victims of CER.

To achieve their maximum potential, biologic products need to be evaluated in real-world situations, not just clinical trials (NEHI 2009), because real-world data can yield different results. Observational CER (OCER) could help fill these evidence gaps (Gliklich 2009). OCER would promote the type of real-world data collection that would capture the nuances among products that biotechnology companies do not want lost to large trials. Opposition to using this type of research for reimbursement decisions includes the lack of an agreed-upon methodology and the increased room for bias. Biotechnology firms may want to consider taking the lead on developing appropriately rigorous OCER studies to prove the validity of observational research.

Partnerships

BIO has joined the recently assembled Partnership to Improve Patient Care (PIPC), a group of stakeholders that have similar interests regarding CER. The coalition's stated goal is to "promote comparative effectiveness research that supports patient access and informed health care decision making and fosters continued medical progress" (PIPC 2009). This group, which also includes physicians, patient advocacy groups, Pharmaceutical Research and Manufacturers of America, and AdvaMed, has outlined distinct goals for CER that it believes are in the best interest of patients. Ultimately, PIPC would like to see CER provide patients and physicians with information they need to make the best medical decisions, improve the quality of care, and continue progress in the medical field. PIPC views the primary focus of CER as an opportunity to disseminate quality information to patients, not a cost-cutting prospect for the government (PIPC 2009).

The biotechnology industry has already made some important choices and taken a few crucial steps concerning its relationship with CER through partnerships with organizations like BIO and PIPC. The industry also should consider conducting head-to-head trials or OCER studies so that its products will fare well under CER evaluations.

References

ARRA (American Recovery and Reinvestment Act of 2009). Pub. L. No. 111-5, §9201.

BIO (Biotechnology Industry Organization). BIO's comments to the Federal Coordinating Council (FCC) on Comparative Effectiveness Research. April 13, 2009. «http://bio.org/ healthcare/compeffective/ 20090413.pdf». Accessed Aug. 26, 2010.

Brownlee S. *Overtreated: Why Too Much Medicine Is Making Us Sicker and Poorer.* New York: Bloomsbury USA. 2007.

Gliklich R, Mack C. Comparative effectiveness research in the real world. *Next Generation Pharmaceutical*. Feb. 7, 2010. «http://www.ngpharma.com/article/Comparative-Effectiveness-Research-in-the-Real-World». Accessed Aug. 26, 2010.

Kahn M. NICE says Bristol Myers' Orencia drug too costly. Reuters UK. Aug. 2, 2007. «http://uk.reuters.com/article/idUKL0150695120070801». Accessed Aug 26, 2010.

NEHI (New England Healthcare Institute). Balancing act: comparative effectiveness research and innovation in U.S. healthcare. April 2009. «http://www.nehi.net/publications/39/balancing_act_comparative_effectiveness_research_and_innovation_in_us_health_care». Accessed Aug 26, 2010.

PIPC (Partnership to Improve Patient Care). «http://www.improvepatientcare.org». Accessed Aug 26, 2010.

Disclosure

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